APPENDIX 1

UNIVERSITY OF WISCONSIN-MADISON

Hope Through Research

Since 1989, when the CF gene mutation was identified on chromosome 7, research to find a cure has increased dramatically. Within one year of the CF gene mutation discovery, researchers were able to correct in the laboratory the basic CFTR defect. The following summaries represent some of the research currently underway to develop a cure for people with CF.

For further information contact a CF Center near you or visit the Research section of the Cystic Fibrosis Foundation.

Activating Alternative Chloride Channels

Since it is known that with CF the chloride channels do not function properly, researchers have been exploring ways to activate alternative chloride channels.

In 1991, researchers administered the drug Uridine Triphosphate, or UTP, by nasal spray to people with CF and observed that the exchange of salts (sodium and chloride) and water in their nasal cells had improved.

Further research is underway to examine the effectiveness of using aerosolized UTP in the lower respiratory tracts of people with CF.

Gene Therapy

Researchers believe that certain viruses may be able to enter the cells that line the respiratory system and correct the genetic material, or the DNA, that causes the chloride channel to malfunction.

Laboratory studies done in the early 1990s showed this method to be successful in correcting the chloride channel of the cells that line the nasal passages of people with CF.

Researchers are now examining different kinds of viruses to determine the most effective use of gene therapy to correct the defective chloride channel in the lungs of people with CF.

Researchers are also looking at ways to use microscopic fat globules to deliver the normal gene to the cells.

Natural Airway Defenses Against Infection

Researchers recently identified defensin, a bacteria-fighting substance in the fluid that lines the airways of people with and without CF. Laboratory studies showed that defensins exposed to high concentrations of salt lost their abilities to destroy bacteria.

Since people with CF have higher levels of salt in their airway fluids, their defensins become inactivated, resulting in an increased risk for lung infections. These findings offer researchers direction for future studies of ways to correct or improve the lung immunity of people with CF.

Novel Nutritional Therapy

Fats, or fatty acids, in the body's cell walls are important for the cell to function normally. People with CF

have been found to be deficient in these fatty acids.

Docosahexaenoic acid, or DHA, is a particular fatty acid that recently has been found to correct the basic defect in the organs affected by CF. In experiments using mice with symptoms of CF, the mice improved when given DHA.

Clinical trials with people who have CF are underway to evaluate the safety and effectiveness of using DHA to treat CF.

Repair Protein Therapy

Laboratory studies of 8-cyclopentyl-1, 3-dipropylxanthine, or CPX, have found that this new drug "repairs" the chloride channel defects found in the delta F508 gene mutation.

Clinical trials with people who have CF are currently underway to examine the safety and effectiveness of this drug, which can be taken orally.

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